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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Reparixin for the prevention of graft loss in pancreatic islet transplantation

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Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

On 27 September 2011, orphan designation (EU/3/11/912) was granted by the European Commission to Dompé S.p.A., Itlay, for reparixin for the prevention of graft rejection in pancreatic islet transplantation.

The sponsorship was transferred to Dompé farmaceutici s.p.a., Italy, in March 2015.

What is graft rejection in pancreatic islet transplantation?

Pancreatic islets are clusters of cells (beta cells) within the pancreas which produce insulin. Pancreatic islet transplantation can be used to treat patients with type I diabetes that is difficult to control.

Graft rejection occurs when the recipient's body rejects the transplanted pancreatic islets (the 'graft'). It is caused by the patient's immune system (the body's natural defenses) recognising the transplanted cells as 'foreign' and reacting against them. This results in inflammation and damage to the graft.

Graft rejection in pancreatic islet transplantation is long-term debilitating because it can lead to the progressive loss of beta cells which can result in failure to cure type I diabetes.

What is the estimated number of patients affected by the condition?

At the time of designation, graft rejection in pancreatic islet transplantation affected less than 0.02 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 1,000 people*,

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 507,700,000 (Eurostat 2011).

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and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What methods of prevention are available?

At the time of designation, no satisfactory methods were authorised in the EU for the prevention of graft loss in pancreatic islet transplantation.

How is this medicine expected to work?

Reparixin blocks the activity of a chemokine called CXCL8. Chemokines are proteins produced by certain cells that act as a chemical signal to attract other cells. CXCL8 is produced by cells of the immune system when they recognise the pancreatic islet graft as foreign. It is an important factor in the graft rejection process, as it sends a signal to other immune cells to move to the site of the graft in order to produce an immune response.

Reparixin is expected to work by attaching to the receptors on the surface of immune cells that CXCL8 normally attaches to, thereby blocking its action and preventing graft rejection.

What is the stage of development of this medicine?

The effects of reparixin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with pancreatic islet transplantation were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for the prevention of graft rejection in pancreatic islet transplantation or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 July 2011 recommending the granting of this designation.

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Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

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For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Reparixin	Prevention of graft rejection in pancreatic islet transplantation
Bulgarian	Репариксин	За предотвратяване на отхвърляне на придсадката при трансплантация на парче от панкреаса
Croatian	Repariksin	Prevencija odbacivanja presatka kod transplantacije otočića pankreasa
Czech	Reparixin	Prevence rejekce štěpu během transplantace Langeransových ostrůvků pankreatu
Danish	Reparixin	Forebyggelse af implantatafstødning ved transplantation af langerhanske øer
Dutch	Reparixin	Preventie van transplantaatafstoting bij transplantatie van de eilandjes van Langerhans.
Estonian	Repariksiin	Implantaadi äratõuke vältimiseks pankrease rakkude siirdamisel
Finnish	Repariksiini	Siirteen hylkimisen esto haiman osittaissiirrossa
French	Réparixine	Prévention du rejet de greffe lors d'une transplantation d'îlots de Langerhans pancréatiques
German	Reparixin	Zur Prävention der Abstoßungsreaktion bei Inselzelltransplantationen
Greek	Ρεπαριξίνη	Για την πρόληψη απόρριψης των μοσχευμάτων κατά τη μεταμόσχευση παγκρεατικών νησίδων
Hungarian	Reparixin	Beültetési kilökődés megakadályozására hasnyálmirigy testecske transzplantációnál
Italian	Reparixin	Prevenzione delle crisi di rigetto dopo trapianto di isole pancreatiche
Latvian	Repariksīns	Transplantāta atgrūšanas novēršanai aizkuņģa dziedzera saliņu transplantācijas laikā
Lithuanian	Repariksinas	Transplantato atmetimo reakcijos prevencija kasos salelių transplantacijos metu
Maltese	Reparixin	Prevenzjoni ta' riġettar ta' l-organu waqt trapjant tal-islets tal-frixa
Polish	Reparyksyn	Zapobieganie odrzuceniu przeszczepu podczas transplantacji wysp trzustkowych
Portuguese	Reparixina	Prevenção da rejeição de enxertos no decurso de transplantes de ilhéus pancreáticos
Romanian	Reparixină	Prevenirea rejetului de grefă în cazul transplantului de celule insulare pancreatice
Slovak	Reparixín	Prevencia odmietnutia štepu pri transplantácii Langerhansových ostrovčekov pankreasu
Slovenian	Repariksin	Preprečevanje zavrnitve presadka med presajanjem pankreasnih otočkov
Spanish	Reparixina	Prevención del rechazo del injerto en el trasplante de islotes pancreáticos

[1] At the time of transfer of sponsorship

Language	Active ingredient	Indication
Swedish	Reparixin	Förebyggande av avstötning vid transplantation av ö-celler från pancreas
Norwegian	Repariksin	Forebygging av transplantatrejeksjon ved transplantasjon av øyceller fra pankreas
Icelandic	Reparixín	Til að koma í veg fyrir höfnun græðlings við ígræðslu briseyja